

## Sales Force Expansion

- Doubled sales force: hiring/training Aug-Oct 2010 (38 to 77 sales reps)
- Newly expanded sales force began call activity Nov 1
  - Significant increase in MS-treating neurologists targeted for sales calls
    - Now also targeting child neurologists for IS sales calls
- MS paid RxS increasing since November
  - November matched previous monthly record
  - December set new record
  - January near previous record level
  - February set new record

## Infantile Spasms

- Devastating, refractory form of childhood epilepsy
  - Very poor developmental outcome if inadequately treated
- Not responsive to standard anti-epileptic drugs
- Ultra-rare orphan disorder
  - 1,500 to 2,000 patients annually
- Typically occurs in children less than 2 years old
  - Characterized by
    - “spasms” -- a specific type of seizure
    - “hypsarrhythmia” -- abnormal EEG pattern

## Acthar and IS

- Used by over half of child neurologists
- FDA approval 10/15/10
  - 7 year orphan exclusivity for IS indication
- Crisis therapy
- Treatment for 2-4 weeks\*
  - In a randomized, single-blinded, controlled study, 87% of patients achieved overall response (no spasms and no hypersarrhythmia) at two weeks versus 29% on prednisone
    - \$100K-\$125K/Rx
    - About half of patients receive drug for free



\*Based on prescription data

## IS Sales Plan

- Significant variability in quarterly prescriptions
- Q4-2010 sales within historic range
  - Promotion effort began 11/1/10
  - Potential to increase IS revenue
    - Acthar currently used to treat 30-50% of IS patients



## Nephrotic Syndrome

- Characterized by excessive spilling of protein from the kidney into the urine (proteinuria)
- Can result in end-stage renal disease (ESRD), dialysis, transplant
- Significant unmet need
  - Few treatment options

## Acthar and NS

- FDA-approved on label indication for reduction of proteinuria in:
  - Idiopathic types of nephrotic syndrome
    - Idiopathic membranous nephropathy
    - Focal segmental glomerular sclerosis (FSGS)
    - IgA nephropathy
  - Lupus nephritis
- Treatment for 4-6 months\*
- \$150K-250K/Rx



QUESTCOR®  
\*Based on prescription data

## Proof of Concept Data

- Available November 2010
- Case series showed response from Acthar in refractory idiopathic membranous nephropathy (on-label)
  - 9 of 11 patients met response criteria
- Positive signal received in diabetic nephropathy investigator initiated trial (not on-label)
  - 9 of 15 patients on Acthar met response criteria and none have required dialysis

## R&D Effort in NS

- Dose response trial for idiopathic membranous nephropathy (on-label)
  - \$5-7M multi-center trial, n~100
  - Reduction of proteinuria is endpoint
- Presently designing a well controlled study in collaboration with FDA for diabetic nephropathy (off-label)
- Proof-of-concept study with different dosing regimens and a placebo
- Objectives will be to determine if Acthar is efficacious and safe in a placebo controlled study
- If successful, next step will be a larger Phase II trial

## NS Business Plan

- Nov 2010 ASN meeting
  - First meeting with commercial team presence
  - Data presented via podium/posters
  - 30+ doctor advisory board meeting
- Hired 5 reps to sell Acthar to nephrologists
  - Develop selling process and generate sales
  - Initiate sales efforts in early March 2011
  - Expand sales force if sales increase
- Peer review publication of case series in March 2011

# Immediate Acthar Growth Opportunities

- MS - Build on sales momentum, lots of market headroom
- IS - Incremental market share growth
- NS - Establish Acthar as a therapeutic alternative in this sizeable market



Represents estimated net sales market opportunity based on internal company estimates

\*\* Represents approximate current net sales annualized run rate based on internal company estimates



## Financials

Profitable

Debt Free

Cash Flow Positive

# 2010 Financial Results

Record Sales (up 30%) and Solid Earnings (EPS up 35%)

	2010	2009
Net Sales (\$M)	\$115.1	\$88.3
Gross Margin	93%	92%
Operating Inc (\$M)	\$53.8	\$41.2
EPS	\$0.54	\$0.40